Current Clinical Program Portfolio Feb-18 Award **Funding** Therapeutic Percent Number, PI, General Disease **General Class** (ICOC Cell (for Cell Time Into Trial Stage Cell Source Rationale **Project Goal** Institution Program Indication Therapeutic Area of Approach Approved) Therapy) Award NEURO THERAPEUTICS Neurologic Disorders: Injuries SP3A-07552 Lebkowski Up to 12,000 Americans suffer a spinal cord injury each year. Leads to a Strategic Oligodendrocyte high level of permanent disability and decreased life expectancy. Asterias Allogeneic oligodendrocyte Safety, Dosing, Efficacy -Biotherapeutics Cell Therapy \$14,323,318 Allogeneic Partnership II Ph 1/2a Spinal Cord Injury progenitors Neurologic Injury Progenitors Currently no approved therapies. motor improvement. Safety and efficacy Stroke is a major cause of long-term disability and there are no proven compared to sham surgery nedical treatments for chronic stroke. Intracerebral delivery of mprovement in motor CLIN2-10344 Clinical Trial Modified bone marrow-derived modified MSCs provides a well tolerated treament with the potential to activity on stroke affected Bates, SanBio Ph 2b mesenchymal stem cells (MSCs) mprove motor function in these patients Stage Project Neurologic Injury Allogeneic Stroke is the leading cause of adult disability. There is no medical CLIN1-09433 Late Stage therapy that promotes stroke recovery. Cells derived from H9 ESC act Steinberg, Preclinical H9 ESC-derived neural stem NSC or NPC (ESC via secretion of paracrine factors to modulate brain repair processes in Stanford Projects IND Ischemic Stroke Cell Therapy \$5,300,000 derived) preclinical stroke models. Neurologic Injury Allogeneic Obtain an active IND Neurologic Disorders: Neurodegenerative Disease Team DR2A-05320, Therapy ALS is a devastating disease with no cure. This cell therapy intends to CLIN2-09284 Development Allogeneic neural progenitor Genetically support sick motor neurons via astrocyte replacement and pro-survival Svendsen, Clinical Trial ALS (Amyotrophic cells genetically modified with Neurodegenerative Modified Cell \$17,842,617 growth factors. Allogeneic neural stem cells, genetically modified to Safety, Dosing, Efficacy -Cedars-Sinai Stage Projects Ph 1/2a lateral sclerosis) GDNF Disorder Therapy \$6.154.067 NSC or NPC express GDNF, injected into the spinal cord, Lower limb strength ALS is a fatal neurodegenerative disease for which there is currently no adequate treatment. Autologous MSCs are propagated ex vivo and Autologous MSCs cultured to induced to secrete neurotrophic factors. NurOwn cells are returned to CLIN2-09894 Clinical Trial ALS (Amyotrophic enhance secretion of growth Veurodegenerative the patients in the target area of damage. Previous trials showed safety Safety and efficacy of three Kern, Brainstorm Stage Projects lateral sclerosis) factors (NurOwn) Disorder Cell Therapy \$15,912,390 MSC Autologous and encouraging signs of efficacy. repeated doses. Eye Disease Age-related macular degeneration is a progressive disease resulting in Duane Roth death of the retinal pigment epithelium (RPE) causing distortion to Disease Tear Allogeneic functionally entral vision and eventually to legal blindness. Incidence - 1:1359 in the Safety. Efficacy - slow Therapy oolarized hESC-derived RPE US. Approach is replacement therapy with viable RPE cells delivered on disease progression, DR3-07438 Development Adult Macular nonolayers on synthetic Cell Therapy, a synthetic membrane mimicking native state with RPE cells on Bruch's maintain and restore visual Ph 1 \$18,922,665 Humayun, USC Degeneration substrate Eye Disease Combination Allogeneic cuity Retinitis pigmentosa (RP) is a progressive retinal degeneration that affects over 1.5 million people worldwide. Unfortunately, treatment is still rather limited. A single sub-retinal injection of human neural progenitor cells offers dramatic preservation of vision. Grafted Cells LSP1-0835 Late Stage survive for an extended period, secrete pro-survival factors and Wang, Cedars-Preclinical Retinitis Subretinal injection of human extracellular matrix, reduce oxidative stress response and preserve \$4,954,514 Sinai Projects IND Pigmentosa neural progenitor cells Eye Disease Cell Therapy NPC Allogeneic vision and RPE integrity. Obtain an active IND Retinitis pigmentosa (RP) is a severe form of blindness that runs in DR2A-05739 Disease Tean families with an incidence of 1:4000. Good target for stem cell therapy Klassen LIC Therapy IND Ph Retinitis Allogeneic retinal progenitor due to the defined loss of specific cells. Proposed mechanism: Rescue Safety and efficacy - visual Irvine Development 1/2a Pigmentosa cells Eye Disease Cell Therapy \$17,306,668 RPC Allogeneic the light sensing photoreceptors. acuity. Safety and efficacy -CLIN2-09698 Clinical Trial Retinitis Allogeneic retinal progenitor Follow-on study based on Phase 1/2a clinical trial. Continue to assess improvement in visual Stage Projects Ph 2b Eye Disease Cell Therapy \$8,295,750 RPC safety and establish efficacy. function at 12 months. Klassen, Jcyte Pigmentosa cells Allogeneic imbal stem cell deficiency results in inability to heal following ocular Cultivated autologous human surface injury leading to corneal opacity. Cultivated autologous limbal Clinical Trial CLIN1-08686 imbal stem cells on human tem cells transplanted back to the patient allow restoration and Stage Projects LSC Deng, UCLA Corneal Blindness amniotic membrane Eye Disease Cell Therapy \$4,244,211 Autologous maintenance of a normal corneal surface Obtain an active IND BLOOD & CANCER THERAPEUTICS Blood Disorders Untreated alpha thalassemia major is almost universally fatal in utero. Current treatment requires in utero blood transfusions and monthly blood transfusions for life or a bone marrow transplant if a suitable donor is identified. The proposed treatment is a maternal bone marrow CLIN2-09183 Clinical Trial Alpha Thalassemia Maternal bone marrow derived transplant in utero that takes advantage of maternal-fetal immune Safety and feasibility, Mackenzie, UCSF Stage Projects Blood Disorder Cell Therapy \$12,131,817 Ph1 Major HSC transplant in utero HSC Allogeneic tolerance, and may provide a definitive cure. efficacy. Lentiviral vector modified CGD prevents white blood cells from killing foreign invaders. Patients autologous CD34+ have persistent, untreatable tissue infections. Affects 1:200,000 in US. X-linked Chronic nematopoietic stem/progenitor Genetically Usually diagnosed before age 5, without treatment children die before Primary: Safety and Efficacy.

Modified Cell

Therapy

\$7,402,549

HSC

Blood Disorder

age 10. Project plan is transplantation of severe X-CGD patients that

Autologous lack matched donors using gene-corrected autologous HSCT.

Secondary: Restoration of

immune function

CLIN2-08231

Kohn UCLA

Clinical Trial

Stage Projects

Ph 1/2

Granulomatous

Disease

cells via transplantation &

engraftment

Award							Funding	Therapeutic				Percent
Number, PI,					General Disease	General Class	(ICOC	Cell (for Cell				Time Into
Institution	Program	Trial Stage	Indication	Therapeutic	Area	of Approach	Approved)	Therapy)	Cell Source	Rationale	Project Goal	Award
										An inherited mutation in the hemoglobin gene causes red blood cells to		
										"sickle" under conditions of low oxygen. Affects 1:500 African-		
	Duane Roth			Autologous HSC, genetically						Americans and is common in Hispanic-Americans. Median survival is 42	D: 0 () () ()	
	Disease Team			corrected ex vivo by lentiviral vector mediated addition of a		Ctill-				years for males and 48 years for females. More than 80% of patients	Primary: Safety, feasibility.	
DR3-06945	Therapy Development			hemoglobin gene that blocks		Genetically Modified Cell				lack an HLA-identical sibling donor. Project plan is genetic correction of adult bone marrow hematopoietic cells by adding a novel therapeutic	Secondary: Hematopoietic Recovery; RBC function;	
Kohn, UCLA	III	Ph 1	Sickle Cell Disease		Blood Disorder	Therapy	\$13,935,441	HSC	Autologous	hemoglobin gene that blocks sickling of the red blood cells.	Quality of life assessment	
ĺ				Autologous HSC, genetically		.,	, , ,			In ADA-SCID allogeneic HSCTs from non-matched sibling donors are a	Primary: Safety. Secondary:	
			ADA-SCID (severe	corrected ex vivo by lentiviral		Genetically				high risk procedure. Efficacy of chronic enzyme replacement therapy is	Efficacy, gene marking,	
CLIN2-09339	Clinical Trial		combined immune	vector mediated addition of		Modified Cell				uncertain in the long-term. Preliminary data indicates that OTL-101 may	immune reconstitution.	
Kohn, UCLA	Stage Projects	Ph2	deficiency)	human ADA gene	Blood Disorder	Therapy	\$20,000,000	HSC	Autologous	significantly improve outcomes compared to available therapies.	Registrational trial.	
CLIN2-09504			X-SCID (X-linked	Autologous HSC, genetically		Genetically				Catastrophic immunodeficiency disorder caused by mutation in IL2RG;	Primary: Safety and feasibility. Secondary:	
Sorrentino, St.	Clinical Trial		severe combined	corrected ex vivo by lentiviral		Modified Cell				Without a curative transplant-based therapy, X-SCID is lethal typically in	Efficacy; gene marking;	
Jude's	Stage Projects	Ph 1/2	immunodeficiency)	vector mediated correction	Blood Disorder	Therapy	\$11,924,780	HSC	Autologous	first year of life.	immune reconstitution	
		,	,,,				, , , , , , ,			.,		
			Conditioning									
			regimen for									
			allogeneic HSC									
	Di T		transplantation for							Monoclonal antibody that targets CD117 and promotes engraftment of	Safety. Dosing. Efficacy - HSC	
DR2A-05365	Disease Team Therapy		SCID (Severe Combined	MAb that depletes endogenous						hematopoietic stem cells. Could replace toxic conditioning regimens and enable chemotherapy-free transplants. Enabled donor cell HSC	engraftment, immune	
Shizuru, Stanford	Development	IND, Ph 1	Immunodeficiency)		Blood Disorder	Biologic	\$19,068,382			engraftment and cure of disease in an animal model of SCID.	reconstitution.	
		,	,,,				7-1/111/111			Primary immune deficiency due to Artemis gene. Most difficult to treat		
			ART-SCID (Artemis-							by allogeneic hematopoietic stem cell transplantation (HSCT) due to		
	Late Stage		deficient severe	Autologous HSC, genetically		Genetically				increased sensitivity to alkylating agents and radiation. Autologous		
CLIN1-08363,	Preclinical		combined	corrected ex vivo by lentiviral		Modified Cell				gene modified HSCT transplantation to overcome allogeneic stem cell		
Puck, UCSF	Projects	IND	immunodeficiency)	vector mediated correction	Blood Disorder	Therapy	4,268,865	HSC	Autologous	transplant difficulty.	Obtain an active IND	
CLIN1-10084, Porteus,	Late Stage Preclinical			Autologous HSC, genetically corrected ex vivo by CRISPR-		Genetically Modified Cell				Gene editing using CRISPR-Cas9 technology has the potential to correct		
Stanford	Projects	IND	Sickle Cell Disease	mediated correction	Blood Disorder	Therapy	5,194,431	HSC	Autologous	the sickle cell mutation.	Obtain an active IND	
HIV/AIDS							., .,					
				Autologous HSC transduced ex								
				vivo with a lentiviral vector								
DR1-06893				engineered to express an		Genetically					Safety. Efficacy - slow	
Symonds,	Discoss Toom I	Dh 1/2a	HIV/AIDS	shRNA against CCR5 & a fusion	LIIV/AIDE	Modified Cell	ćo 270 722	usc	Autologous	Cal-1 increases the number of HIV-protected cells in the body. Uses	disease progression,	
Calimmune	Disease Team I	Ph 1/2a	HIV/AIDS	inhibitor. Gene modified HSCs via a	HIV/AIDS	Therapy	\$8,278,722	HSC	Autologous	shRNA to CCR5 and C46 to confer cellular resistance to HIV infection.	mitigate need for ART.	
				lentiviral vector that encodes a						Lentiviral vector encodes a triple combination of HIV-resistance genes		
				triple combination of HIV-		Genetically				and a pre-selective marker. Vector transduced CD34+ cells will safely	Safety. Efficacy - immune	
CTS1-08231	Clinical Trial			resistance genes and a tCD25		Modified Cell				engraft, divide and differentiate in vivo into mature myeloid and	reconstitution, viral load and	
Abedi, UC Davis	Stage Projects	Ph 1	HIV/AIDS	pre-selective marker	HIV/AIDS	Therapy	\$7,402,549	HSC	Autologous	lymphoid cells.	HIV status.	
			1							Autologous hematopoietic stem cells gene edited ex vivo to eliminate		
SP3A-07536	Ctuat:-		1	Autologous UCCtiU		Genetically Modified Cell				expression of HIV entry co-receptor CCR5. Cells carrying disrupted CCR5	Cofoty Efficac:	
Zaia, City of Hope	Strategic Partnership III	Ph 1	HIV/AIDS	Autologous HSCs genetically modified to disrupt CCR5	HIV/AIDS		\$5,583,438	HSC	Autologous		Safety. Efficacy - engraftment.	
Hope Hematologic Cand		LUI	HIV/AID3	mounted to disrupt CCR5	HIV/AID3	Therapy	35,205,436	пэс	Autologous	icens.	engratunent.	
											Safety. Dosing. Follow on	
	Duane Roth		1							Cancer is a leading cause of death in CA. Many cancers resist current	trials will include other	
	Disease Team		1							therapies due to therapy-resistant cancer stem cells (CSCs). Discovered	cancers and will test	
	Therapy		1	Monoclonal antibody (anti-						a protein, ROR1, present on CSCs but not on normal healthy cells.	cirmtuzumab alone or in	
DR3-06924	Development			ROR1) targeting CLL cancer	Hematologic		44.4=				combination with other anti-	
Kipps, UCSD	III	Ph 1	CLL	stem cells	Malignancy	Biologic	\$4,179,600			plan is to treat chronic lymphocytic leukemia with cirmtuzumab.	cancer therapies.	
			1							Cancer is a leading cause of death in CA. Many cancers resist current therapies due to therapy-resistant cancer stem cells (CSCs). Discovered		
										a protein, ROR1, present on CSCs but not on normal healthy cells.		
ı		1		Monoclonal antibody (anti-						Developed an antibody, cirmtuzumab, that is specific for ROR1. Project		
CLIN2-10192	Clinical Trial			ROR1), combined with tyrosine	Hematologic					plan is to treat chronic lymphocytic leukemia or mantle cell carcinoma	Evaluate dosing and	

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Motion of an open control of the con									Evpanded CD24+				
CPUID-1976 CPU													
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Sing-Projects 90 ordinary improves monitored evaluate incl. Molganeses Old Harvay Scientific and Monitorial Conference of the Conference o		Clinical Trial		_		Hematologic			_		· ·		
Facilities Collection Col			IND	-		_	Cell Therapy	\$3,797,117		Allogeneic		Obtain an active IND	
Leganose COSA- Mathematic and Mathe				, .			.,						
Company Comp									Expanded CD34+				
Semantiange de managegeriet cette au de l'angeriet de l'an									stem and		appropriate preparation called myeloablation. The endothelial cells		
Fire register. Fire Applications Fire Application					Matched cord blood derived				progenitor cells		used in the co-culture are thought to aid the engraftment of the stem		
Application Critical Trial (Singerice) Dirical Trial (Singerice) System Process (F) 130 and Numbers of supplications (Coll Therroy) 5500000000000000000000000000000000000	CLIN2-10386			Hematologic	hematopoietic stem and				from cord blood		and progenitor cells into the bone marrow via secretion of angiocrine		
Rockshare Region Prigints Region	Finnegan,			malignancies	progenitor cells expanded by co-				and gene-		factors. The remainder of the cord blood cells in the cell product also		
Add 25 are invented to traps and fill only the turner concer cells and popular concercions and popular	Angiocrine	Clinical Trial		including leukemia	culture with genetically	Hematologic			modified		aid in the engraftment as well as provide anti-viral and anti-bacterial		
Section Company Comp	Bioscience	Stage Projects	Ph 1b	and lymphoma	modified endothelial cells cells.	Malignancies	Cell Therapy	\$5,000,000	endothelial cells	Allogeneic		Safety.	
CURS-16776													
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Delawer, Northal Stage Projects Ph 2 AML Umbilled cord blood stem cells Malignancy Cell Therapy 5,522.105 Cord blood Molecnee Cell The reverse chemotherapy for AML			IND	AIVIL	DNA binding payload.		conjugate (ADC)	\$6,863,755				Obtain an active IND	
CIN2-20144 Clinical Trial Choo, 27/82 Slege Projects Ph 130 AMM only and Ch47 monocloral antibody International Chinical Trial Choo, 27/82 Slege Projects Ph 130 AMM only and Ch47 monocloral antibody International Chinical Trial Choo, 27/82 Slege Projects Ph 130 AMM only antibody in the control of the cont			Dh 2	A N A I	Umbilical cord blood stom colls	_	Coll Thorany	\$6.022.100	Cord blood	Allogopoic			
CIN2-10144 Clinical Trial Clinical Trial Anni-CD47 monodonal antiblody Iternatiologic Clinical Trial Clinical T	Delatiey, Notila	Stage Projects	FILE	AIVIL	Offibilical cord blood sterri cells.	ivialigitaticy	Cell Therapy	30,322,103	Cora biooa	Allogeneic			
LIN2-30144 Clinical Trial Chao, 47th C Sage Projects Ph 1b AML Anti-CD47 monoclonal antibody with azoitotime AML AML AML AML AML ANTI-CD47 monoclonal antibody with azoitotime AML													
LCUR2-10144 Clinical Trial Chapter (Page 1) B AMI. Anti-CD47 monocloral antibody (Page Projects of Chapter (Page 1) B AMI. Anti-CD47 monocloral antibody (Page Projects of Chapter (Page Projects of Cha													
Cho, Affine Stage Projects Ph 10 AML with sachted virus protection of Cell Trial Publisher, CHAS Stage Projects Ph 17 Virus Infection Specific Trolls Publisher, CHAS Stage Projects Ph 17 Virus Infection Specific Trolls Interval Publisher, CHAS Stage Projects Ph 17 Virus Infection Specific Trolls Interval Publisher, CHAS Stage Projects Ph 17 Virus Infection Specific Trolls Interval Publisher, CHAS Stage Projects Ph 17 Virus Infection Specific Trolls Interval Publisher, CHAS Stage Projects Ph 10 Virus Infection Specific Trolls Interval Publisher Stage Projects Ph 10 Virus Infection Specific Trolls Interval Publisher Stage Projects Ph 10 Virus Infection Specific Trolls Interval Publisher Stage Projects Ph 10 Virus Interval Publisher Stage Proj	CUN2-10144	Clinical Trial			Anti-CD47 monoclonal antibody	Hematologic					-		
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Publisher, CHA Stage Projects Ph 1/2 Virol Infection Project Ph 1/2 Virol Infection Project Ph 1/2 Virol Infection Ph 1/2 Virol Infec								40,000,000					
CUN2-05577	Pulsipher, CHLA		Ph 1/2	Viral infection	1		Cell Therapy	\$4,825,587	T Cell	Allogeneic			
Disease Team Disea			,				, ,	, ,, ,,					
Disease Feam Disease Feam Disease Feam Disease Feam Disease Feam Therapy Rubss, UCLA Development IND, Ph 1 Disease Feam Therapy Draw Forth Disease Feam Disease Feam Disease Feam Heard Private College Colleg	Spear, Poseida	Stage Projects	Ph 1	Multiple myeloma	CAR-T	Malignancy	Cell Therapy	\$19,997,927	CAR-T	Autologous			
Disease Team Disea	Solid Cancers												
DB2A-05309 Therapy Ribas, UCA Development (ND, Ph 1 Ovaria) annit tumor T cell receptor. Solid Tumor Therapy S19,999,563 HSC Autologous system cells to produce a continual supply of the immune and successful tumor are the most prevalent form of cancer, and are a major cause of death worldwide. The mean all molecule being developed inhibitor targeting serine/hierance is small molecule with tumor cell ineas and cancer stem. Cell Side tumor cell search worldwide. The mean and cancer stem cells incompleted in tumor cell search worldwide. The mean and cancer stem cells incompleted in tumor cell search worldwide. The mean and cancer stem cells incompleted in tumor cell search worldwide. The mean and cancer stem cells and prevent the search worldwide. The mean and cancer stem cells and prevent the search worldwide. The mean and cancer stem cells and prevent the search worldwide. The mean and cancer stem cells and prevent the search worldwide. The mean and cancer stem cells and prevent the search worldwide. The mean and cancer stem cells and prevent the search worldwide. The mean and cancer stem cells and prevent the search worldwide. The mean and cancer stem cells and prevent the search worldwide. The mean and cancer stem cells and prevent the search worldwide. The mean and cancer stem cells and prevent the search worldwide and the search worldwide. The mean and cancer stem cells and prevent the search worldwide. The mean and cancer stem cells and prevent the search worldwide and the				Advanced tumors							There are few options for patients whose cancers have metastasized		
Ribas, UCLA Development Disease Team Diseas		Disease Team			_							feasibility. Secondary:	
Duane Roth Disease Team Therapy Disease Team Therapy Disease Team Therapy Development Slamon, UCLA III Ph 1 Solid Tumor Therapy Development Slamon, UCLA III Ph 1 Solid Tumor Therapy Development Slamon, UCLA III Ph 2 Solid Tumor Therapy Development Slamon, UCLA III Ph 3 Solid Tumor Therapy Development Slamon, UCLA III Ph 3 Solid Tumor Therapy Development Slamon, UCLA III Ph 3 Solid Tumor Therapy Development Slamon, UCLA III Ph 3 Solid Tumor Therapy Development Slamon, UCLA III Ph 3 Solid Tumor Therapy Development Slamon, UCLA III Ph 3 Solid Tumor Therapy Solid Tumor Small Molecule S6,524,317 Therapy Development Slamon, UCLA III Ph 4 Solid Tumor Therapy Solid Tumor Small Molecule S6,524,317 Therapy Small Molecule					F '							_	
Disease Feam Therapy Development Therapy Development Display the properties of the protection of the p	Ribas, UCLA		IND, Ph 1	Ovarian)	an anti-tumor T cell receptor.	Solid Tumor	Therapy	\$19,999,563	HSC	Autologous			
DR3-07067 Development III Ph 1 Solid Tumor Salmon, UCLA III Ph 1 Solid Tumor S													
DR3-07067 Development Slamon, UCLA III Ph 1 Solid Tumor cells and cancer stem cells Solid Tumor by hagocytic macrophages by delivering a potent don't can the ending of the target size of the ending of the end													
Slamon, UCLA III Ph 1 Solid Tumor cells and cancer stem cells Solid Tumor small Molecule \$6,924,317 regrowth after treatment. CLOP4 is overexpressed on cancer and cancer stem cells and prevents their elimination by phagocytic macrophages by delivering a potent "don't eat me" signal. Hu599-G4 is a humanized monoclonal antibody (mAb) that binds to CD47 and blocks its interaction with its receptor, thereby enabling phagocytosis of cancer cells. Anti-CD47 is highly synergistic incombination with other anti-cancer therapies including objective response rate (ORR) CLIN2-09577 Clinical Trial Chao, 47 inc Stage Projects Ph1b/2 Solid Tumor Solid Tumor Biologic S10,234,048 Ab unmortargeting mAbs such as cetuximab. CLIN2-10248 Clinical Trial Brown, COH Stage Projects Ph 1 Malignant Giloma cancer stem cells Solid Tumor Therapy S12,753,854 CAR-T Autologous expressing tumor cells ORGAN SYSTEMS THERAPEUTICS Brown Corrections of the control of		1											
CLIN2-09577 Clinical Trial Chao, 47inc Stage Projects Ph1b/2 Solid Tumor Biologic S10,234,048 Abtumor-targeting mAbs such as cetuximab													
their elimination by phagocytic macrophages by delivering a potent "don't eat me" signal. Hu59-G4 is a humanized monoclonal antibody (mAb) that binds to CA7 and blocks its interaction with its receptor, thereby enabling phagocytosis of cancer cells. Anti-CD47 is highly synergistic in combination with other anti-cancer therapies including Chao, 47Inc Stage Projects Ph1b/2 Solid Tumor + cetux/mab Solid Tumor Biologic \$10,234,048 Abtumor-targeting mAbs such as cetux/mab. CLIN2-10248 Clinical Trial Brown, COH Stage Projects Ph 1 Malignant Glioma cancer stem cells Solid Tumor Therapy S12,753,854 CAR-T Autologous expressing tumor cells ORGAN SYSTEMS THERAPEUTICS Bone Disease Team Disea	Slamon, UCLA		Ph 1	Solid Tumor	cells and cancer stem cells	Solid Tumor	Small Molecule	\$6,924,317			S .	cancers.	
"don't eat me" signal. Hu5F9-G4 is a humanized monoclonal antibody (mAb) that binds to CD47 and blocks its interaction with its receptor, thereby enabling phagocytosis of cancer cells. Anti-CD47 monoclonal antibody synergistic in combination with other anti-cancer therapies including tumor-targeting mAbs such as cetuximab. CLIN2-09577 Clinical Trial Chao, 47Inc Stage Projects Ph1b/2 Solid Tumor + cetuximab Solid Tumor Biologic S10,234,048 Ab	1 '	1											
CLIN2-09577 Clinical Trial Chao, 47Inc Stage Projects Ph 1b/2 Solid Tumor + cetuximab Solid Tumor Biologic \$10,234,048 Ab	1 '	1									,, , , , , , , , , , , , , , , , , , , ,		
CLIN2-09577 Clinical Trial Chao, 47inc Stage Projects Ph1b/2 Solid Tumor + cetuximab Solid Tumor Biologic S10,234,048 Abtumor-targeting mAbs such as cetuximath. (ORR) CLIN2-10248 Clinical Trial Brown, COH Stage Projects Ph 1 Malignant Glioma Cancer stem cells Solid Tumor Therapy S12,753,854 CAR-T Autologous expressing tumor cells ORGAN SYSTEMS THERAPEUTICS Bone Disorders Femoral head osteonecrosis (aka avascular necrosis) is a disease caused by loss of blood supply to the bone, leading to be nece ideath, end stage hip arthitis and total hip replacement. There is an unment eneed for treatment of this disease, that affects individuals at prime of life (peak age 35 years). This small molecule therapeutic recruits bone forming cells to site of damage, where they serve the dual function of laying DR2A-05302 Therapy endogenous bone marrow	1	1									-		
CLIN2-09577 Clinical Trial Stage Projects Ph1b/2 Solid Tumor Ph1b/2 So	1 '	1										Safety Dosing Efficacy	
Chao, 47Inc Stage Projects Ph1b/2 Solid Tumor + cetuximab Solid Tumor Biologic \$10,234,048 Ab tumor-targeting mAbs such as cetuximab. (ORR) Genetically Genetically Genetically Modified Cell Therapy S12,753,854 CAR-T Autologous expressing tumor cells of treatment of this disease, that affects individuals at prime of life (peak age 35 years). This small molecule therapeutic recruits bone forming of endogenous bone marrow Solid Tumor Therapy Biologic Solid Tumor Therapy S12,753,854 CAR-T Autologous expressing tumor cells Solid Tumor Therapy S12,753,854 CAR-T Autologous expressing tumor cells Solid Tumor Solid Tumor Therapy S12,753,854 CAR-T Autologous expressing tumor cells Solid Tumor Solid Tumor Therapy S12,753,854 CAR-T Autologous expressing tumor cells Solid Tumor Solid Tum	CLIN2-09577	Clinical Trial			Anti-CD47 monoclonal antibody						,		
Gliobastoma (GBM) is lethal with 5 year survival rate is only 5.5%. CAR-T are "living drug" with potential to actively seek out and destroy malignant cells. This study clinical study will investigate two different course of local delivery of CAR-T cells to target and eliminate IL13R persistence, biodistribution and biological activity ORGAN SYSTEMS THERAPEUTICS Bone Disorders Femoral head osteonecrosis (aka avascular necrosis) is a disease caused by loss of blood supply to the bone, leading to bone cell death, end stage hip arthritis and total hip replacement. There is an unmet need for treatment of this disease, that affects individuals at prime of life (peak age 35 years). This small molecule therapeutic recruits bone forming Disease Team Disease Team Disease Team DR2A-05302 Therapy Gliobastoma (GBM) is lethal with 5 year survival rate is only 5.5%. CAR-T are "living drug" with potential to actively seek out and destroy malignant cells. This study clinical study will investigate two different croutes of local delivery of CAR-T cells to target and eliminate IL13R persistence, biodistribution and biological activity Safety, Feasibility, Persistence, biodistribution and biological activity and biological activity Department of this disease, that affects individuals are prime of life (peak age 35 years). This small molecule therapeutic recruits bone forming cells to site of damage, where they serve the dual function of laying PD effects on bone turnover, biomarkers. Determine PD endogenous bone marrow			Ph1h/2	Solid Tumor	·	Solid Tumor	Biologic	\$10.234 048	Ah		, ,		
CLIN2-10248 Clinical Trial Brown, COH Stage Projects Ph 1 Malignant Glioma cancer stem cells Solid Tumor Therapy \$12,753,854 CAR-T Autologous Surpressing tumor cells by loss of blood supply to the bone, leading to bone cell death, end stage hip arthritis and total hip replacement. There is an ummet need for treatment of this disease, that affects individuals at prime of life (peak age 35 years). This small molecule therapeutic recruits bone forming Disease Team Disease Team Disease Team Draza-05302 Therapy endogenous bone marrow endogenous endo	5.135, 471116	- Luge : rojects		30 1411101		55 1411101	2.0.0510	710,204,040	, 10	 -		()	
CLIN2-10248 Clinical Trial Brown, COH Stage Projects Ph 1 Malignant Glioma cancer stem cells Solid Tumor Therapy \$12,753,854 CAR-T Autologous expressing tumor cells to target and eliminate IL13R persistence, biodistribution and biological activity CAR-T Autologous Ph 1 Malignant Glioma Ph 1 Malignant Glioma Cancer stem cells Solid Tumor Therapy \$12,753,854 CAR-T Autologous Expressing tumor cells Autologous Expressing tumor cells Solid Tumor Therapy Solid Tumor Therap	1 '	1									, , ,		
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Brown, COH Stage Projects Ph 1 Malignant Glioma cancer stem cells Solid Tumor Therapy \$12,753,854 CAR-T Autologous expressing tumor cells and biological activity ORGAN SYSTEMS THERAPEUTICS	CLIN2-10248	Clinical Trial			T cells engineered to target						, ,		
Bone Disorders Femoral head osteonecrosis (aka avascular necrosis) is a disease caused by loss of blood supply to the bone, leading to bone cell death, end stage hip arthritis and total hip replacement. There is an unment need for treatment of this disease, that affects individuals at prime of life (peak age 35 years). This small molecule therapeutic recruits bone forming Disease Team to enhance homing of cells to site of damage, where they serve the dual function of laying PD effects on bone turnover, endogenous bone marrow down new bone, and stimulating revascularization to prevent further biomarkers. Determine			Ph 1	Malignant Glioma		Solid Tumor		\$12,753,854	CAR-T	Autologous			
Femoral head osteonecrosis (aka avascular necrosis) is a disease caused by loss of blood supply to the bone, leading to bone cell death, end stage hip arthritis and total hip replacement. There is an unmet need for treatment of this disease, that affects individuals at prime of life (peak age 35 years). This small molecule therapeutic recruits bone forming Disease Team to enhance homing of cells to site of damage, where they serve the dual function of laying PD effects on bone turnover, endogenous bone marrow down new bone, and stimulating revascularization to prevent further biomarkers. Determine										,			
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stage hip arthritis and total hip replacement. There is an unmet need for treatment of this disease, that affects individuals at prime of life (peak age 35 years). This small molecule therapeutic recruits bone forming to enhance homing of eells to site of damage, where they serve the dual function of laying DR2A-05302 Therapy Synthetic molecule, LLP2A-Ale, to enhance PK. Determine PC. Dete	1 '	1									· · · · · · · · · · · · · · · · · · ·		
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Synthetic molecule, LLP2A-Ale, to enhance homing of DR2A-05302 Therapy Synthetic molecule, LLP2A-Ale, to enhance homing of endogenous bone marrow Synthetic molecule, LLP2A-Ale, to enhance homing of endogenous bone marrow Disease Team down new bone, and stimulating revascularization to prevent further biomarkers. Determine	1 '	1									9 1	Safety, tolerability.	
Disease Team DR2A-05302 Disease Team Therapy Drace to enhance homing of endogenous bone marrow DR2A-05302 Disease Team to enhance homing of cells to site of damage, where they serve the dual function of laying down new bone, and stimulating revascularization to prevent further biomarkers. Determine	1	1			Synthetic molecule, LLP2A-Ale,						The state of the s		
DR2A-05302 Therapy endogenous bone marrow down new bone, and stimulating revascularization to prevent further biomarkers. Determine	1	Disease Team											
	DR2A-05302				endogenous bone marrow								
Lane, UC Davis Development Ph 1 a/b Osteonecrosis MSCs to bone surface Bone Disorder Small Molecule \$19,999,867 bone cell death. immunogenicity.	Lane, UC Davis	Development	Ph 1 a/b	Osteonecrosis	MSCs to bone surface	Bone Disorder	Small Molecule	\$19,999,867			bone cell death.	immunogenicity.	

Award							Funding	Therapeutic				Percent
Number, PI,					General Disease	General Class	(ICOC	Cell (for Cell				Time Into
Institution	Program	Trial Stage	Indication	Therapeutic	Area	of Approach	Approved)	Therapy)	Cell Source	Rationale	Project Goal	Award
Cartilage Disorde					7.1.52			,,,	1	1	,	
CLIN1-09472												
Wang, Cellular	Late Stage											
Biomedicine	Preclinical			Allogeneic adipose-derived								
Group	Projects	IND	Osteoarthritis	MSCs	Cartilage Disorder	Cell Therapy	\$2,291,976	MSC	Allogeneic		Obtain an active IND	
				Small molecule injected intra-								
				articularly that promotes resident cartilage mesenchymal								
CLIN1-08309			Osteoarthritis,	stem cell differentiation into								
Schultz, Calibr	CLIN1	IND	cartilage injuries	chondrocytes	Cartilage Disorder	Small Molecule	\$1,667,832				File an IND	
			Ů,	Small molecule injected intra-			, , , , , , ,					
				articularly that promotes								
				resident cartilage mesenchymal								
CLIN2-10388			Osteoarthritis,	stem cell differentiation into								
Sahelijo, Calibr	CLIN2	Ph 1	cartilage injuries	chondrocytes	Cartilage Disorder	Small Molecule	\$8,447,523					
Cardiovascular &	Vascular Disord	lers		1	I	I	ı		T T		I	
											Primary: Determine whether	
											treatment is safe and causes	
											reduction in cardiac scar size	
											in patients with heart failure	
			Heart dysfunction								after a heart attack.	
DR2A-05735	Disease Team		after myocardial							Heart failure is a progressive disease with a high risk of mortality.	Secondary: Assess for other	
Smith, Capricor	Therapy		infarction/Chronic	Allogeneic cardiosphere derived						Cardiosphere-derived cells (CDCs) reduce scar size after heart attack in	structural or functional	
Inc.	Development	Ph 2	heart failure	cells	Disease	Cell Therapy	\$19,782,136	CDC	Allogeneic	preclinical animal models and in a prior clinical trial.	cardiac benefits.	
										Pulmonary arterial hypertension (PAH) is a progressive condition with	Daines and Coffee Consendence	
CLIN2-09444										no cure, survival is poor. Cardiosphere-derived cells (CDCs) decrease wall thickening of lung small blood vessels in preclinical studies.	Primary: Safety. Secondary: Exploratory efficacy	
Lewis, Cedars-	Clinical Trial		Pulmonary Arterial	Allogeneic cardiosphere derived						Improvement in lung blood vessels is expected to reduce cardiac right	measures of right ventricular	
Sinai	Stage Projects	Ph1a/b	Hypertension	cells	Vascular Disease	Cell Therapy	\$7,354,772	CDC	Allogeneic	ventricular dysfunction.	function.	
	,		,,							,		
											Primary: Safety and	
			Duchenne							Heart failure is a leading cause of death for Duchenne muscular	tolerability in DMD patients.	
CLIN2-08334			muscular							dystrophy patients. Cardiosphere-derived cells (CDCs) decrease	Secondary: Structural or	
Ascheim,	Clinical Trial		dystrophy	Allogeneic cardiosphere derived						myocardial fibrosis, improve cardiac function and induce regeneration	functional cardiac benefits,	
Capricor, Inc.	Stage Projects Disease Team	Ph 2	cardiomyopathy	cells	Disorder	Cell Therapy	\$3,376,259	CDC	Allogeneic	of heart muscle in preclinical models of DMD.	quality of life improvements.	:
DR2A-05394	Therapy		Ischemic heart	Allogeneic hESC-derived	Cardiovascular					5.7 million Americans suffer from heart failure, and the end stage 2 year survival rate is 50%. hESC-CM promote new blood vessel formation and	Obtain an active IND for a first-in-human trial in heart	
Wu, Stanford	Development	IND	failure	cardiomyocytes	Disease	Cell Therapy	\$19,060,330	CM	Allogeneic	improve cardiac function in preclinical models of heart failure.	failure patients.	
Diabetes & Comp			ranare	caraiomyocytes	Discuse	oen merupy	\$13,000,000	C.V.	7 mogenero	improve durate randion in predimed models of near classics	ranare patients.	
										Diabetes mellitus affects 370 million people worldwide.		
										Disproportionately affects certain minority groups and the elderly.		
										Current therapy is self-administration of insulin. Diabetes costs in CA are		
										tens of billions of dollars each year. Directed differentiation of		
401.00				Allogeneic hESC-derived						embryonic stem cells to pancreatic precursor cells. Project plan is		
AP1-08039	Accolorate-	Comparahili		pancreatic cell progenitors in a		Coll Thorson:		Pancreatic		transplantation of pancreatic precursor cells that generate functional islet tissue in vivo that can respond to insulin levels in a more	Drimany Cafoty Casanda	
Foyt, ViaCyte Inc.	Accelerated Pathway I	Comparabili ty Trial	Diabetes: Type 1	device implanted subcutaneously	Endocrine Disorder	Cell Therapy, Combination	\$16,603,160	endocrine progenitor	Allogeneic	physiological manner than direct insulin replacement.	Primary: Safety. Secondary: Efficacy.	
IIIC.	i attiway i	cy iiidi	Siddetes, Type I	Subcutaneously	Endocrine District	COMBINATION	y10,003,100	progenitor	Anogeneic	Children with T1D face lifelong struggles with glycemic control and,	cucy.	
										despite careful management, an increased risk of severe complications.		
										No therapy that maintains or restores pancreatic beta islet cell function		
										is currently		
CLIN2-09730										approved. Evidence indicates that		
Losordo,	Clinical Trial			Autologous ex vivo expanded		,		_		regulatory T-cells (T-regs) maintain immune balance at least in part by	Primary: Safety. Secondary:	
Caladrius	Stage Projects	Ph 2	Diabetes: Type 1	polyclonal regulatory T cells	Endocrine Disorder	Cell Therapy	\$12,211,255	T-reg	Autologous	control of differentiation of multipotent progenitor/stem cells.	Efficacy.	
				hESC-derived pancreatic						There are over 100,000 people in the US with type 1 diabetes so severe		
CLIN1-08671,				progenitor cells delivered in a device that allows direct				Pancreatic		that they are at constant risk of hospitalization and/or death. Within months after administration, this product could provide a source of		
D'Amour,	Clinical Trial			vascularization of the cell		Cell Therapy,		endocrine		insulin producing beta cells to restore those patients' blood sugar to	Obtain an active IND and	
Viacyte	Stage Projects	IND	Diabetes: Type 1	therapy	Endocrine Disorder	Combination	\$3,984,164	progenitor	Allogeneic	normal healthy levels and save their lives.	trial start up	
-,			,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,	hESC-derived pancreatic			, , =	10	3	There are over 100,000 people in the US with type 1 diabetes so severe		
				progenitor cells delivered in a						that they are at constant risk of hospitalization and/or death. Within		
				device that allows direct				Pancreatic		months after administration, this product could provide a source of		
CLIN2-09672,	Clinical Trial			vascularization of the cell		Cell Therapy,		endocrine		insulin producing beta cells to restore those patients' blood sugar to	Primary:Safety and	
	Stage Projects	Ph 1/2	Diabetes: Type 1	therapy	Endocrine Disorder	Combination	\$20,000,000	progenitor	Allogeneic	normal healthy levels and save their lives.	Tolerability	
Kidney Disorders												

Award							Funding	Therapeutic				Percent
Number, PI,					General Disease	General Class	(ICOC	Cell (for Cell				Time Into
Institution	Program	Trial Stage	Indication	Therapeutic	Area	of Approach	Approved)	Therapy)	Cell Source	Rationale	Project Goal	Award
				A Human Acellular Vessel in								
				Patients Needing Renal						Synthetic vascular access grafts for hemodialysis in kidney patients are	Primary: Safety and	
				Replacement Therapy: A						associated with thrombosis, infection and abandonment. Human	tolerability, rate of patency	
CLIN2-08938,				Comparison with ePTFE Grafts						Acellular Vessel (HAV) is made of extracellular matrix from human	of the graft and rate of	
Lawson,	Clinical Trial			as Conduits for Hemodialysis						smooth muscle cells, similar in composition and structure to native	interventions needed to	
Humacyte, Inc.	Stage Projects	Ph 3	Renal dialysis	(HUMANITY)	Endocrine Disorder	Device	\$9,999,528		Allogeneic	tissue.	restore patency.	
										Synthetic vascular access grafts for hemodialysis in kidney patients are		
										associated with thrombosis, infection and abandonment. Human		
CLIN2-09688,				A Human Acellular Vessel in						Acellular Vessel (HAV) is made of extracellular matrix from human		
Lawson,	Clinical Trial			Patients Needing Renal						smooth muscle cells, similar in composition and structure to native	A Comparison with AV	
Humacyte, Inc.	Stage Projects	Ph 3	Renal dialysis	Replacement Therapy.	Endocrine Disorder	Device	\$14,082,865		Allogeneic	tissue.	Fistula	
										Unmet medical need for allogeneic kidney transplants. Need to		
										eliminate chronic rejection/allograft nephropathy that causes gradual		
CLIN2-09439				Donor CD34+ and CD3+ T cells						loss of kidney (50% of graft loss by 12-15 years in HLA mismatched		
Strober,	Clinical Trial		Transplant	for immune tolerance to HLA	Immune tolerance,					recipients). Eliminate the lifelong need for anti-rejection drugs that have	Primary: Safety. Secondary:	
Stanford	Stage Projects	Ph 1	tolerance	mismatched kidney donors.	transplant	Cell Therapy	\$5,069,674	HSC	Allogeneic	numerous cumulative side effects.	Preliminary efficacy.	